Essay

The Royal Society of Medicine

Journal of the Royal Society of Medicine; 2015, Vol. 108(8) 299–303
DOI: 10.1177/0141076815579896

Putting patient-reported outcomes on the 'Big Data Road Map'

Melanie Calvert[1], Rob Thwaites[2], Derek Kyte[1] and Nancy Devlin[3]
[1]University of Birmingham, Birmingham B15 2TT, UK
[2]Takeda Development Centre Europe Ltd, London WC2B 4AE, UK
[3]Office of Health Economics, London SW1E 6QT, UK
Corresponding author: Melanie Calvert. Email: m.calvert@bham.ac.uk

Introduction

The term 'big data' is used to describe large, complex datasets and the associated advances in technology and analytics.[1,2] Within healthcare, this covers many different types of data including, for example: routinely collected clinic data in electronic health records; healthcare claims data, genomics data; public data releases, such as aggregated population health data or clinical trials results; data from automated sensors and smart devices; and information from healthcare forums and social media. In late 2013, the Association of the British Pharmaceutical Industry published its 'Big data road map', which sets out an action plan to direct progress in the use of big data over the next four years.[1] The road map highlights the need to increase awareness and understanding of big data, build capability and capacity, create sustainable data ecosystems and accelerate high-value opportunities. These suggestions do not just apply to pharma, however, but also to healthcare more generally as clinicians, patients and policy-makers look to take advantage of the potential benefits of big data.[3]

There is, however, a risk that patient-reported outcome (PRO) data may be underrepresented in the big data 'revolution'. PRO data are important in providing patients' perspective on their own health. However, in contrast to countries such as the US and Netherlands,[4] PROs are not routinely collected in UK clinical settings (other than for a small number of procedures, via the NHS PROMs programme).[5] If this situation is not addressed, we risk continuing our overreliance on routinely collected clinical indicators to provide evidence for health policy: a potential backwards step in the move towards patient-centred care.

Potential benefits of big data

Big data analytics offer a range of exciting opportunities, including the facilitation and acceleration of targeted drug design, the development of diagnostic algorithms, a shift towards stratified medicine.[1] Big data can provide 'real-world' post-licensing safety data, assist comparative effectiveness research and inform predictive impact modelling. All of these initiatives have the potential to lead to significant patient benefits, through newer and more targeted treatments and provision of information to promote shared decision-making. Increasing amounts and improved access to electronic healthcare data, coupled with advances in analytic methods and computational power and an increased need for 'real-world' evidence of effectiveness, are driving the rapid growth in this field.[6] Collaboration between healthcare providers, academics and the pharmaceutical industry will be essential to maximise the use of big data to inform healthcare policy and delivery.[7]

PROs and big data

PROs are collected using questionnaires providing a systematic way of measuring patients' subjective views about their own health. In trials, PROs provide additional 'patient-centred' data which are unique in capturing the patient's own opinion on the impact of their disease, and its treatment, on their life.[8] This is important: evidence suggests clinical indicators, when used in isolation, can underestimate the impact of a disease upon the individual and overestimate the effectiveness of healthcare interventions.[9] PRO trial data may be used to inform clinical care and decision-making, predict long-term outcomes and influence health policy such as the appraisal of new health technologies by the National Institute for Health and Care Excellence.[10] The use of PROs in clinical trials is well established, but widespread use in routine UK clinical practice is relatively uncommon; where it does take place, data collection and reporting tends to have a focus on provider performance rather than individual patient care.[11]

Routinely collected secondary healthcare data have traditionally concentrated on clinical outcomes such as rates of postsurgical mortality, readmission to hospital, reoperation, infection and serious adverse events. Thus, we have readily accessible big data on these clinically relevant, but relatively rare, safety outcomes.[12] In addition, we have at our fingertips millions of linked primary and secondary care records including information on medical history, diagnostic test results and treatment, however, these records generally lack PRO information. It is important that this situation be addressed, in order that we unlock the potential benefits of big PRO data in the future. These benefits may be realised in enhanced clinical audit activity, in the delivery of tailored patient care and in the facilitation of clinically important pragmatic trials (Box 1). For instance, routine assessment of PROs may be used to guide patient care:

- At a population level. Where PRO data may be used as an outcome in prognostic models to predict those patients most likely to obtain health gains and at an individual level to identify those who are a priority for care.[13]
- At an individual level. Where real-time monitoring of PRO data may help with early identification of problems requiring a prompt response (PRO-Alerts), facilitate improved communication between patients and their clinical team and allow rapid referral to appropriate specialist care when necessary.[14]

What are the opportunities for 'Big PRO Data' and how does this work in practice?

PROs provide us with a unique and far-reaching opportunity to drive healthcare reform (Box 1); however, this requires timely integration into electronic systems and a thoughtful evidence-based approach to their use. In the first instance, we should ensure that big datasets include PROs that are acceptable to a range of stakeholders, the most important of which are patients. This may necessitate the development of 'core outcome sets including PROs' for routine clinical care.[15] PROs should also be rigorously selected based on the rationale for their use, ensuring that they are valid and reliable for the population of interest. Appropriately selected instruments have potential to provide data for more than one purpose but need to be piloted and reviewed to ensure that they are feasible, do not adversely affect the clinical workflow and are cost-effective.[16] For example, routine electronic capture of the EQ-5D across a range of settings may help facilitate cost-effectiveness analyses both pre- and post-marketing launch and provide auditable data. Large-scale implementation needs to be supported with appropriate guidance and formal evaluation. While traditional paper-based questionnaires continue to be used in a number of settings the future undoubtedly will lie with electronic data capture. Technological advancement such as patient portals and mobile phone applications may facilitate advancement in the integration of PROs with electronic health records.[17,18] For instance, a current UK-led initiative is working to link routinely collected electronic PRO data provided by cancer survivors, to cancer registry data, in order to inform risk stratification.[19]

In addition, social media and health forums have been identified as potential sources of evidence regarding patient views that may be used to inform commercial strategies.[20] The use of these complex unstructured data to inform health policy is the subject of considerable debate.[21] Further research will be necessary to develop and refine methods to utilise social media data effectively.

Challenges for big PRO data

The current fragmented approach to PRO data collection in routine care represents a major challenge to the use of PRO data in big datasets.[22] Standardised collection would facilitate pooling of data across providers and facilitate evidence synthesis. It would also allow comparison across healthcare providers, giving valuable information on patient outcomes both to patients and referring GPs (who may use this data to inform decisions when choosing healthcare providers) and commissioners. Harmonisation is required both within and across institutions with collaboration between academia and industry, patient advocates, regulatory bodies, commissioners and policy-makers.[23]

The NHS made laudable progress towards the routine collection of PRO data in healthcare settings with the introduction of the PROMs programme in 2009 – but subsequent progress rolling this out beyond the four elective surgical procedures which were its initial focus has been limited. Furthermore, the means by which data are collected and analysed do not facilitate the real-time use of individual patients' responses in shared decision-making with clinicians. There are technical and other challenges in further developing the PROMs programme so that the data can be used both by and for patients as well as, in more aggregated forms, by budget holders and policy-makers and others making

Box 1. Potential uses of PRO data.

Big PRO data in:	Opportunities for 'Big PRO Data'
Patient care	Routine assessment of PROs may also be used to guide individual care. Real-time monitoring of PRO data may:
	• help with early identification of problems (PRO-Alerts)
	• facilitate communication between patients and their clinical team and
	• allow rapid referral to appropriate specialist care when necessary
Population health management	Expansion of the NHS PROMs initiative across a range of chronic conditions would enable patient perspectives to contribute to the NHS by:
	• 'informing the choices patients make with regard to their treatment and its providers
	• measuring and benchmarking the performance of health care providers
	• linking the payment received by providers to their performance in improving patient health
	• understanding and managing referral from primary to secondary care
	• facilitating co-operation between clinicians and managers in the delivery of care
	• enabling health care professionals to monitor and improve health care practices
	• regulating for safety and quality in health care services'[12]
	At a population level PRO data may be used as an outcome in prognostic models to predict those patients most likely to obtain health gains and to identify those who are a priority for care.
Research	Routine collection of PROs would enhance pragmatic real-world trials conducted using EHRs, by:
	• providing additional, complementary, PRO data through the EHR system
	• reducing the need for additional interventions to collect data from patients
	Routine collection of PROs would also enrich observational research, both cross-sectional and longitudinal, by introducing information and measures from the patient perspective

decisions that affect patients. This includes selection of measures with appropriate psychometric properties for use at an individual patient and group level that have cultural validity and are available in a range of languages. Acceptability of measures that are not over-burdensome will also be crucial. Attempts to standardise measures for use across patient groups (e.g. Patient-reported Outcomes Measurement Information System), use of appropriate methods for instrument development (item response theory) and computerised adaptive testing may go some way to address this.[24]

A key challenge to be faced in the use of PROs for research, audit and to inform individualised care is ensuring that patients understand who has access to their data and, crucially, how this information will be acted upon.[25] Patients' PRO data can sometimes reveal 'worrying levels of psychological distress or physical symptoms that may require an immediate response', known as a 'PRO alert'.[25] For PRO data collected in the clinical care setting, there should be a plan in place to monitor for alerts and an outline of the response options available to healthcare staff. PRO alerts may lead to increased healthcare utilisation; however, they also have the potential to bring cost savings if care can be tailored to those in need. Where PRO data are collected for research purposes, there is debate over whether the responsibility for PRO alert management rests with the research team or with the participant's regular clinical care providers. Such issues should be addressed during the research design phase and require close collaboration between researchers and clinicians. Whatever the PRO alert management plan in operation, both patients and study participants should be fully informed of how their PRO data will be handled.

Increased assessment of PRO data may risk over-medicalisation. Working in partnership with patients and having a clearly defined rationale for assessment will be key to successful integration of PROs in big data. Expansion in the use of PROs will also require the active support and involvement of doctors, nurses, pharmacists, funders and others involved. Convincing examples of the impact of bringing PROs into routine consultations and care, together with standard approaches and technology, will be needed to persuade healthcare professionals of the overall merit of bringing in PROs.

Conclusion

The rapid acceleration in electronic data capture and analytic techniques means that we are at the brink of healthcare revolution. Big PROs data offer exciting possibilities, but we need to do more to facilitate increased routine PRO collection in clinical care for the benefits to be realised. In order to maintain and further develop a patient-centred approach to care, we require the rapid standardised introduction of PROs within electronic health records. This will necessitate a coordinated approach, guided by input from a range of stakeholders, to ensure a clear rationale for PRO assessment utilising optimal instruments, aimed at facilitating high-quality data capture and appropriate governance.[16] Adding PROs to the heart of the big data agenda will open up an important new opportunity in the progress to patient-centred care.

Declarations

Competing interests: We declare the following interests: DK declares no conflicts of interest. MC is a member of the International Society for Quality of Life Research Board of Directors and has received consultancy fees from the pharmaceutical industry for advice on PROs. RT is Chair of the ABPI Pharmaceutical Industry Health Information group and contributed to the development of the ABPI Big Data Road Map. ND is an advisor to the NHS England PROMs programme. The Office of Health Economics receives. The funding from ABPI which has an interest in big data. ND is the elected Chair of the EuroQol Group. The EuroQol EQ-5D is a widely used measure of patient-reported health and is used in the NHS PROMs programme.

Funding: DK is supported by a National Institute for Health Research School for Primary Care Research funded PhD studentship. The funder had no role in the decision to publish or preparation of the manuscript.

Ethical approval: Not applicable

Guarantor: MC

Contributorship: This paper was conceived by MC and RT following the Big Data in Pharma Conference, London, May 2014. MC, DK and ND have expertise in PROs and outcomes methodology. ND has worked on the NHS PROMS Initiative. RT is Chair of the Association of the British Pharmaceutical Industry (ABPI) Pharmaceutical Industry Health Information group and contributed to the development of the ABPI Big Data Road Map. MC wrote the first draft of the manuscript. All authors edited the manuscript each adding important intellectual content. All authors read and approved the final version.

Acknowledgements: None

Provenance: Not commissioned; editorial review (including response to peer review comments from a previous submission to another journal).

References

1. ABPI. Big data road map. *ABPI* 2013.
2. Weber G, Mandl K and Kohane I. Finding the missing link for big biomedical data. *JAMA* 2014; 311: 2479–2480.
3. HM-Government. Personalised Health and Care 2020 Using Data and Technology to Transform Outcomes for Patients and Citizens, 2014. See https://wwwgovuk/government/uploads/system/uploads/attachment_data/file/384650/NIB_Reportpdf (last checked February 2015).
4. Van Der Wees PJ, Nijhuis-Van Der Sanden MW, Ayanian JZ, Black N, Westert GP and Schneider EC. Integrating the use of patient-reported outcomes for both clinical practice and performance measurement: views of experts from 3 countries. *Milbank Quart* 2014; 92: 754–775.
5. NHS England Patient-reported Outcome Measures. See http://www.nhs.uk/NHSEngland/thenhs/records/proms/Pages/aboutproms.aspx (last checked 19 August 2014).
6. Wilson M. The use of ''Big Data'' in health care decisions: where are we and what does the future hold? See http://www.ispor.org/meetings/montreal0614/presentations/First-Plenary-Marcus-Wilson.pdf (last checked 23 June 2014).
7. Jain SH, Rosenblatt M and Duke J. Is big data the new frontier for Academic-Industry Collaboration? *JAMA* 2014; 311: 2171–2172.
8. Calvert MJ and Freemantle N. Use of health-related quality of life in prescribing research. Part 1: why evaluate health-related quality of life?. *J Clin Pharm Ther* 2003; 28: 513–521.
9. Pakhomov S, Jacobsen SJ, Chute CG and Roger VL. Agreement between patient-reported symptoms and their documentation in the medical record. *Am J Managed Care* 2008; 14: 530.
10. NICE. Guide to the methods of technology appraisal 2013. 2013. See http://www.nice.org.uk/Article/PMG9/chapter/the-reference-case.
11. Black N. Patient reported outcome measures could help transform healthcare. *BMJ (Clin Res Ed)* 2013; 346: f167 (last checked March 2015).
12. Devlin NJ and Appleby J. Getting the most out of Proms-Putting health outcomes at the heart of NHS decision-making. *The Kings Fund* 2010. Available at: http://www.kingsfund.org.uk/sites/files/kf/Getting-the-most-out-of-PROMs-Nancy-Devlin-John-Appleby-Kings-Fund-March-2010.pdf (last checked March 2015).

13. Gotay CC, Kawamoto CT, Bottomley A and Efficace F. The prognostic significance of patient-reported outcomes in cancer clinical trials. *J Clin Oncol* 2008; 26: 1355–1363.
14. Andikyan V, Rezk Y, Einstein MH, Gaultiere G, Leitao MM Jr, Sonoda Y, et al. A prospective study of the feasibility and acceptability of a Web-based, electronic patient-reported outcome system in assessing patient recovery after major gynecologic cancer surgery. *Gynecol Oncol* 2012; 127: 273–277.
15. Macefield R, Jacobs M, Korfage I, Nicklin J, Whistance RN, Brookes ST, et al. Developing core outcomes sets: methods for identifying and including patient-reported outcomes (PROs). *Trials* 2014; 15: 49.
16. Wu AW, Kharrazi H, Boulware LE and Snyder CF. Measure once, cut twice—adding patient-reported outcome measures to the electronic health record for comparative effectiveness research. *J Clin Epidemiol* 2013; 66: S12–S20.
17. McCann L, Maguire R, Miller M and Kearney N. Patients' perceptions and experiences of using a mobile phone-based advanced symptom management system (ASyMS©) to monitor and manage chemotherapy related toxicity. *Eur J Cancer Care* 2009; 18: 156–164.
18. Ammenwerth E, Schnell-Inderst P and Hoerbst A. The impact of electronic patient portals on patient care: a systematic review of controlled trials. *J Med Internet Res* 2012; 14e162.
19. Ashley L, Jones H, Velikova G, Thomas J, Brown J, Morris E, et al. Collecting patient reported outcome measures (PROMs) and linking with clinical cancer registry data: preliminary results from feasibility testing of a novel, UK-scalable electronic system (ePOCS). *Psycho-oncology* 2013; 22(Suppl. 1): 1–29.
20. Sawyer J. Maximizing patient reported outcomes through the Internet. *Applied Clinical Trials* (last checked 3 August 3).
21. Rothman M, Gnanaskathy A, Wicks P and Papadopoulos EJ. Can we use social media to support content validity of patient-reported outcome instruments in medical product development? *Value Health J Int Soc Pharmacoecon Outcomes Res* 2015; 18: 1–4.
22. Curtis L. Comparison of Machine Learning & Traditional Outcomes Research Methods from a Researcher's Perspective. Conference: ISPOR 19th Annual International Meeting, 31 May–4 June 2014. URL: http://www.ispor.org/Event/Program/2014Montreal?day=3 (last checked June 2014).
23. Wu AW, Bradford AN, Velanovich V, Sprangers MA, Brundage M and Snyder C. Clinician's checklist for reading and using an article about patient-reported outcomes. *Mayo Clin Proc* 2014; 89: 653–661.
24. Cella D, Riley W, Stone A, Rothrock N, Reeve B, Yount S, et al. The Patient-Reported Outcomes Measurement Information System (PROMIS) developed and tested its first wave of adult self-reported health outcome item banks: 2005–2008. *J Clin Epidemiol* 2010; 63: 1179–1194.
25. Kyte D, Draper H and Calvert M. Patient-reported outcome alerts: ethical and logistical considerations in clinical trials. *JAMA* 2013; 310: 1229–1230.